March 14, 2007

Re: Docket Nos.2006N—0061
and 2006N—0062

Dear Sir or Madam:

The undersigned groups write to endorse the two proposed rules referenced above. One of these proposals would clarify and expand the opportunities for sponsors to establish expanded access programs, and the other clarifies the circumstances in which sponsors may charge for investigational drugs. These proposed rules are valuable additions to the body of regulation governing access to unapproved therapies for patients with serious or life-threatening diseases and may be especially important to pediatric cancer patients in light of the relative paucity of approved pediatric cancer therapies.

The proposal on expanded access programs is particularly welcome. Cancer patient advocates have long sought more access to investigational therapies for those patients who are not eligible for participation in clinical trials. The proposed rule offers important guidance to sponsors who may wish to initiate expanded access programs, and indicates to providers and patients the limits of such programs. There is reason to believe that the proposal will create new access to unapproved therapies without jeopardizing patient participation in clinical trials. While we support expanded access programs for unapproved drugs, we remain convinced that the best avenue for access to new therapies is through the usual regulatory approval process dependent on the results of well designed and executed clinical investigations.

The proposed rule on charging for investigational drugs is also important. The Food and Drug Administration (FDA) is providing much-needed specificity regarding limits on charging for investigational drugs, which will help to restrain companies that seek to commercialize their products prematurely, in advance of full approval based on completion of adequate and well-controlled clinical trials. At the same time, certain investigator-initiated trials exploring new uses of approved drugs may be facilitated by relaxation of the rules governing charging for drugs used in such trials.

We support both of the referenced proposed rules and encourage FDA to adopt them as final regulations.

Sincerely,

American Society of Clinical Oncology
American Society of Pediatric Hematology/Oncology
Association of Pediatric Hematology/Oncology Nurses
Association of Pediatric Oncology Social Workers
Cancer Research and Prevention Foundation/HopeStreet Kids
Children's Brain Tumor Foundation
Children's Oncology Group
CureSearch National Childhood Cancer Foundation
National Children's Cancer Society